Title: To amend the Federal Food, Drug, and Cosmetic Act to establish a time-limited conditional approval pathway, subject to specific obligations, for certain drugs and biological products, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Promising Pathway Act 2.0".

SEC. 2. CONDITIONAL APPROVAL OF NEW HUMAN DRUGS FOR INDIVIDUALS WITH RARE, PROGRESSIVE, AND SERIOUS DISEASES.

(a) In General.—Subchapter A of chapter V of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 351 et seq.) is amended by adding at the end of the following:

"SEC. 524C. CONDITIONAL APPROVAL OF HUMAN DRUGS FOR INDIVIDUALS WITH RARE, PROGRESSIVE, AND SERIOUS DISEASES.

"(a) Conditional Approval; Priority Review; Other Designations.—

"(1) IN GENERAL.—The sponsor of a drug may file with the Secretary an application for conditional approval of an eligible drug described in subsection (b). The Secretary shall approve or deny such application in accordance with subsection (c).

"(2) PRIORITY REVIEW.—The Secretary shall give priority review to an application for conditional approval of an eligible drug described in subsection (b).

"(3) OTHER DESIGNATIONS.—If a drug that is granted conditional approval under this section is eligible for a special designation by the Secretary under this Act, including as a drug for a rare disease or condition under section 526, all applicable benefits of such other designation shall be available for use under such conditional approval, including any tax credits and waiving of fees under chapter VII.

"(4) OTHER PROGRAMS.—A sponsor of a drug seeking conditional approval of such drug under this section may also seek designation, exclusivity, or approval, as applicable, of such drug under other applicable provisions of this Act or the Public Health Service Act, subject to the requirements of such provisions.

"(b) Eligibility.—

"(1) IN GENERAL.—A drug may be eligible for conditional approval under this section if such drug is intended to treat a disease or condition that is—

"(A) rapidly progressive, terminal, and has substantial unmet medical need, as determined by the Secretary; or

"(B) a rare disease or condition (as defined in section 526(a)(2)) that results in a substantially shortened lifespan, substantial reduction in quality of life, or other substantial adverse health effects, as determined by the Secretary.

"(2) EXCLUSION FROM ELIGIBILITY.—A drug that is intended to treat or respond to a material threat identified by the Secretary of Homeland Security under section 319F–2(c)(2)(A)(ii) shall not be eligible for conditional approval under this section.

"(c) Standard of Review for Conditional Approval.—

"(1) REQUIREMENTS.—The Secretary shall only approve an application for conditional approval of a drug under this section if—

"(A) the Secretary determines that—

"(i)(I) evidence of safety for the drug has been established by—

"(aa) the completion of a phase 1 clinical investigation of the drug (as described in section 312.21 of title 21, Code of Federal Regulations (or successor regulations)); or

"(bb) another demonstration of safety, as determined appropriate by the Secretary; and

"(II) evidence of effectiveness in treating a given indication (which indication is congruent with the eligibility requirements of subsection (b)), as established by an ongoing or completed phase 2 clinical investigation of the drug (as described in section 312.21 of title 21, Code of Federal Regulations (or successor regulations)); or

"(ii) in the case of a drug that is intended to treat a terminal pediatric rare disease or condition (as defined in section 526(a)(2)) that does not predominately affect adults—

"(I) evidence of safety for the drug has been established in accordance with clause (i)(I); and

"(II) the drug shows preliminary evidence of clinical effectiveness based upon studies in animal models; and

"(B) the sponsor has provided a written affirmation of the sponsor's intent to pursue under section 505 of this Act or section 351 of the Public Health Service Act approval of the drug, which affirmation shall include a justification and a plan for pursuing such approval.

"(2) ROLLING, REAL-TIME REVIEW.—

"(A) IN GENERAL.—If the Secretary determines, after preliminary evaluation of data submitted by the sponsor, that a drug may meet the standard for conditional approval, the sponsor may submit portions of an application for conditional approval of a drug under this section for evaluation by the Secretary before the sponsor submits a complete application, which submission shall include—

"(i) a schedule for submission of information necessary to make the application complete; and

"(ii) a payment of any fee that may be required under section 736.

"(B) REVIEW.—The Secretary—

"(i) shall evaluate each application submitted under subparagraph (A) to assess whether such application is complete or ready to be filed; and

"(ii) may commence review of portions of such application for approval.

"(3) USE OF REAL-WORLD EVIDENCE.—

"(A) IN GENERAL.—The Secretary shall allow the use of real world evidence (as defined in section 505F(b)), including real world data used to generate real world evidence, and of external sources of data, including prospective or retrospective natural history data, to support an application for conditional approval under this section.

"(B) DATA INTEGRITY REQUIREMENTS.—In using evidence described in subparagraph (A) to support an application for conditional approval under this section, the sponsor shall consider the guidance of the Food and Drug Administration entitled 'Data Standards for Drug and Biological Product Submissions Containing Real-World Data' and dated December 2023 (or successor guidance).

"(d) FDA Authority to Withdraw Conditional Approval.—

"(1) IN GENERAL.—The Secretary may withdraw the conditional approval of a drug under this section if—

"(A) after adequate review of appropriate safety data, including data from an observational registry established under subsection (g), the Secretary determines that such data no longer supports conditional approval;

"(B) the Secretary determines that the application for conditional approval submitted under subsection (a)(1) contained an untrue statement of material fact; or

"(C) the Secretary determines that the drug is no longer eligible under subsection (b).

"(2) FDA EXAMINATION AUTHORITY.—

"(A) IN GENERAL.—For purposes determining whether to withdraw the conditional approval of a drug under paragraph (1), the Secretary may—

"(i) review any available clinical data made available through clinical trials or an observational registry under subsection (g), applicable to such drug; and

"(ii) determine whether the sponsor of such drug is in violation of a requirement established under paragraph (3) or (4) of section 505(o) or section 505–1 with respect to the drug.

"(B) TRANSPARENCY.—

"(i) IN GENERAL.—The Secretary may require drug sponsors and observational registries under subsection (g) to submit the data described in subparagraph (A) for the purposes of the review under that subparagraph.

"(ii) FINES.—The Secretary may levy fines on sponsors and observational registries that do not comply with a request for data under clause (i) within such

reasonable timeframe as is established by the Secretary.

"(3) EFFECT OF WITHDRAWAL.—

"(A) AVAILABILITY TO NEW PATIENTS.—

"(i) IN GENERAL.—If a conditional approval is withdrawn under this subsection, the sponsor may not make the drug available to any new patients, but may continue to make such drug available to patients who started taking the drug prior to the date of withdrawal.

"(ii) EFFECT.—Nothing in this subparagraph shall be construed to require—

"(I) a patient to continue taking a conditionally approved drug if such patient decides to stop taking such drug; or

"(II) the sponsor to ensure such drug continues to be manufactured after the date of withdrawal.

"(B) CIVIL MONETARY PENALTY.—Any sponsor who makes available to new patients a drug for which conditional approval has been withdrawn under this subsection shall be subject to such civil monetary penalty as is determined by the Secretary.

"(4) WITHDRAWAL NOTICE.—Upon determining to withdraw the conditional approval of a drug under paragraph (1), the Secretary shall submit written notice to the sponsor of such drug and such withdrawal shall be effective on the date that is 14 days after the date of such submission of notice.

"(5) APPEALS.—Not later than 180 days after the date of enactment of the Promising Pathway Act 2.0, the Secretary, by rule, shall establish a process by which a sponsor of a drug for which conditional approval was withdrawn under paragraph (1) may appeal such withdrawal.

"(6) AUTOMATIC WITHDRAWAL.—

"(A) IN GENERAL.—If the sponsor of a drug that receives conditional approval under this section does not submit an application for renewal of such conditional approval under subsection (f)(2) by the deadline under that subsection, such conditional approval shall automatically be withdrawn in accordance with paragraph (3) on the date on which such conditional approval expires.

"(B) MARKETING REQUIREMENT.—If any drug that receives conditional approval under this section is not brought to market within 1 year of the date on which the conditional approval is granted, such conditional approval, along with any benefits described in subsection (a)(3), shall automatically be withdrawn in accordance with paragraph (3) on such date.

"(C) NO RIGHT TO APPEAL; EFFECT OF AUTOMATIC WITHDRAWAL.—

"(i) IN GENERAL.—A sponsor shall not have the right to appeal an automatic withdrawal under this paragraph.

"(ii) EFFECT.—The Secretary shall have no means or power to prevent an automatic withdrawal under this paragraph from occurring.

"(e) Labeling; Review of Materials.—

"(1) IN GENERAL.—Sponsors may not make available to patients a drug conditionally approved under this section, unless—

"(A) all labeling and advertising of such drug contains the statement 'conditionally approved for a limited population' in a prominent manner and adjacent to, and not more prominent than—

"(i) the proprietary name of such drug, if any; or

"(ii) if there is no proprietary name, the established name of such drug, if any, as defined in section 502(e)(3), or, in the case of a drug that is a biological product, the proper name, as defined by regulation; and

"(B) the prescribing information for the drug required by section 201.57 of title 21, Code of Federal Regulations (or any successor regulation) includes the following statement: 'This drug is conditionally approved for use in a limited and specific population. This drug has not received full approval by the Food and Drug Administration. Conditional approval of this drug may be withdrawn at short notice.'.

"(2) SUBMISSION.—Not later than 45 days before such materials are distributed, all promotional, educational, and marketing materials for such drug shall be submitted to the Secretary for review.

"(3) PUBLIC LIST.—The Secretary shall maintain a list of all drugs conditionally approved under this section on a publicly accessible website. Such website shall briefly describe what each conditionally approved drugs is and list the 1 or more diseases or conditions for which the drug is indicated.

"(f) Renewal of Conditional Approval; Requirement to Bring Drug to Market.—

"(1) DURATION; RENEWALS.—The conditional approval for a drug under this section is effective for a 2-year period. The sponsor may request renewal of such conditional approval for up to 3 subsequent 2-year periods. Conditional approval with respect to a drug shall not exceed a total of 8 years from the initial date the drug was granted conditional approval.

"(2) APPLICATIONS FOR RENEWAL OF CONDITIONAL APPROVAL.—

"(A) IN GENERAL.—Except as provided in subparagraph (C), the sponsor of a drug seeking a renewal of conditional approval for such drug under this subsection shall submit to the Secretary, not later than 180 days before the date on which such conditional approval expires, an application that contains the applicable information described in paragraph (3) in a standardized format determined by the Secretary.

"(B) PROCESS FOR GRANTING RENEWALS.—Not later than 180 days after the date of enactment of the Promising Pathway Act 2.0, the Secretary, by rule, shall establish the process for granting a renewal under this subsection.

"(C) EXEMPTION FOR SMALL POPULATION DISEASES.—

"(i) IN GENERAL.—The Secretary shall exempt from the requirements of subparagraph (A) and paragraph (3) an application for a renewal of conditional approval for a drug under this subsection if the Secretary determines that the

population affected by the disease or condition that the drug is intended to treat does not support additional preliminary evidence of effectiveness (as defined in paragraph (3)(D)).

"(ii) APPLICATION FOR EXEMPTION.—Sponsors may submit an application for exemption under this subparagraph not later than 180 days before the date on which the conditional approval expires.

"(iii) APPLICATION PROCESS.—Not later than 180 days after the date of enactment of the Promising Pathway Act 2.0, the Secretary shall establish a standardized application process for purposes of this subparagraph.

"(iv) DEADLINE.—The Secretary shall approve or deny an application under this subparagraph before the date on which the conditional approval expires.

"(v) APPEALS.—Not later than 180 days after the date of enactment of the Promising Pathway Act 2.0, the Secretary shall establish process under which a sponsor may appeal a denial of an application under this subparagraph.

"(3) ADDITIONAL PRELIMINARY EVIDENCE OF EFFECTIVENESS.—The information described in this paragraph is the following:

"(A) FOR THE FIRST APPROVAL RENEWAL.—With respect to an application under paragraph (2) for the first renewal of conditional approval for a drug under this subsection, additional preliminary evidence of effectiveness of the drug, as compared to the evidence provided in the initial application for conditional approval for the drug under subsection (c).

"(B) FOR THE SECOND APPROVAL RENEWAL.—With respect to an application under paragraph (2) for the second renewal of conditional approval for a drug under this subsection, additional preliminary evidence of effectiveness of the drug, as compared to the evidence provided in the renewal application described in subparagraph (A).

"(C) FOR THE FINAL APPROVAL RENEWAL.—With respect to an application under paragraph (2) for the third renewal of conditional approval for a drug under this subsection, a written affirmation from the head of the drug's review division of the Office of New Drugs or the Office of Therapeutic Products asserting that a third renewal is necessary—

"(i) for patients who have benefitted from such drug to retain access to such drug; and

"(ii) to generate additional preliminary evidence of effectiveness for the purposes of attaining approval under section 505 of this Act or section 351 of the Public Health Service Act.

"(D) DEFINITION.—In this paragraph, the term 'preliminary evidence of effectiveness' means—

"(i) clinical evidence generated by an ongoing or completed clinical trial conducted in accordance with section 11.22 of title 42, Code of Federal Regulations (or successor regulations);

"(ii) real-world evidence (as defined in section 505F(b)); or

"(iii) evidence from an observational registry under subsection (g).

"(4) DENIAL OF RENEWAL ON THE BASIS OF DATA FRAUD.—The Secretary may deny the application for renewal of conditional approval for a drug under this subsection if the Secretary, in conducting a review under subsection (d)(2), finds that the evidence provided in such application under subparagraph (A) or (B) of paragraph (3) was fraudulently manipulated by the applicable observational registry and that such application substantially relies on such data.

"(g) Observational Registries.—

"(1) ESTABLISHMENT.—

"(A) IN GENERAL.—Subject to subparagraph (C), the sponsor of a drug conditionally approved under this section shall establish an observational registry, for patients who are or will be treated with such drug, that pertains to the disease or condition that the drug is intended to treat.

"(B) REGISTRIES.—In establishing an observational registry for a drug under subparagraph (A), the sponsor may—

"(i) establish a new observational registry;

"(ii) use an existing observational registry that pertains to the disease or condition such drug is intended to treat;

"(iii) combine 1 or more existing observational registries that pertain to the disease or condition such drug is intended to treat with a new observational registry; or

"(iv) combine 2 or more existing observational registries that pertain to the disease or condition such drug is intended to treat.

"(C) APPROVAL OF REGISTRY AND RIGHT TO APPEAL.—Not later than 180 days after the date of enactment of the Promising Pathway Act 2.0, the Secretary shall establish—

"(i) a process to approve or deny the establishment of an observational registry under subparagraph (A); and

"(ii) a process for sponsors that received such a denial to appeal the denial.

"(2) REQUIREMENT FOR PATIENTS TO ENROLL IN OBSERVATIONAL REGISTRY.—

"(A) IN GENERAL.—A drug conditionally approved under this section shall not be made available to a patient unless such patient is enrolled in the applicable observational registry described in paragraph (1).

"(B) INFORMED CONSENT.—

"(i) IN GENERAL.—Prior to enrolling in an observational registry under subparagraph (A), a patient shall provide informed consent in accordance with clause (ii).

"(ii) APPLICATION OF CERTAIN REQUIREMENTS.—The requirements for informed consent under part 50 of subchapter A of chapter I of title 21, Code of

Federal Regulations (or successor regulations), shall apply to enrollment an observational registry under this paragraph.

"(3) SUBMISSION OF PATIENT DATA.—

"(A) IN GENERAL.—The sponsor of a drug conditionally approved under this section shall be responsible for obtaining and submitting patient data to the applicable observational registry described in paragraph (1).

"(B) SUBMISSION STANDARDS.—Not later than 180 days after date of enactment of the Promising Pathway Act 2.0, the Secretary shall establish data submission standards for sponsors to comply with for purposes of subparagraph (A) to ensure that registry data is consistent and clinically informed.

"(4) REQUIREMENTS FOR REGISTRIES.—An observational registry described in paragraph (1) for a drug conditionally approved under this section may be operated by the sponsor of such drug or, at the sponsor's discretion, a third party, for-profit organization, or nonprofit organization.

"(5) RISK AND BENEFIT DATA.—

"(A) IN GENERAL.—The sponsor of a drug conditionally approved under this section shall submit relevant risk and benefit data to the applicable observational registry described in paragraph (1).

"(B) ONLINE PORTAL.—The Secretary shall operate an online portal on an existing website of the Secretary for sponsors to submit data described in subparagraph (A).

"(6) ACCESSIBILITY.—

"(A) IN GENERAL.—An observational registry described in paragraph (1) shall—

"(i) not later than 30 days after receipt of a request, provide patients (or their designated representatives) with access to such patient's personal registry information; and

"(ii) provide approved researchers and medical professionals access to de-identified and aggregated data from the registry for the purposes of indication- and disease-specific and translational research into conditions and diseases relating to the disease or condition that the drug tracked by the observational registry is intended to treat.

"(B) APPROVED RESEARCHERS AND MEDICAL PROFESSIONALS.—Not later than 180 days after the date of enactment of the Promising Pathway Act 2.0, the Secretary, by rule, shall establish a process for approving researchers and medical professionals for purposes of subparagraph (A)(ii).

"(7) EFFECT.—Nothing in this section shall be construed to modify or limit the Secretary's authority to require for a drug conditionally approved under this section any type of postapproval study under any other provision of law, including sections 505(o)(3), 505B, and 506.

"(h) Pursuit of a Different Indication.—

"(1) IN GENERAL.—In the case of a drug conditionally approved under this section for

which such approval was withdrawn under subsection (d), expired under subsection (f)(1), or was denied for renewal under subsection (f)(4), not later than 2 years after the date of withdrawal, expiration, or denial, as applicable, the sponsor of such drug shall have the opportunity to petition the Secretary to receive conditional approval of such drug, in accordance with this section, for a different indication.

"(2) PROCESS.—Not later than 180 days after the date of enactment of the Promising Pathway Act 2.0, the Secretary shall establish a process for petitions under paragraph (1).

"(i) Transition to Other Forms of Approval.—

"(1) IN GENERAL.—A drug that receives conditional approval under this section may be granted approval under section 505 of this Act or section 351 of the Public Health Service Act during the period in which such conditional approval is in effect. Effective on the date on which approval for such drug is granted under section 505 of this Act or section 351 of the Public Health Service Act, such conditional approval shall be automatically withdrawn in accordance with subsection (d)(3).

"(2) CONSIDERATION OF CERTAIN EVIDENCE.—In determining whether to approve under section 505 of this Act or section 351 of the Public Health Service Act a drug that has received conditional approval under this section, the Secretary may consider evidence from the observational registry for the drug under subsection (g).

"(j) Informed Consent.—

"(1) IN GENERAL.—Prior to being prescribed a drug conditionally approved under this section, a patient shall provide informed consent in accordance with paragraph (2).

"(2) APPLICATION OF CERTAIN REQUIREMENTS.—The requirements for informed consent under part 50 of subchapter A of chapter I of title 21, Code of Federal Regulations (or successor regulations), shall apply to drugs conditionally approved under this section.

"(3) OBSERVATIONAL REGISTRIES.—An observational registry established for a drug in accordance with subsection (g) may obtain, and maintain records of, informed consent of a patient on behalf of the drug sponsor, in accordance with paragraph (2).

"(4) COMMON RULE.—Drugs conditionally approved under this section shall comply with subpart A of part 46 of title 45, Code of Federal Regulations (commonly known as the 'Common Rule') (or successor regulations), if applicable.

"(k) Limitation on Liability.—With respect to any claim under State law relating to a drug made available pursuant to a grant of conditional approval under this section, no liability shall lie against a sponsor or manufacturer of the drug, or any health care provider who prescribes or administers the drug, absent intentional wrongdoing.

"(l) Report to Congress.—

"(1) IN GENERAL.—Not later than 2 years after the date of enactment of the Promising Pathway Act 2.0, and once every 2 years thereafter, the Secretary, in collaboration with drug sponsors, shall submit a report to Congress on all drugs granted conditional approval under this section. Such report shall include—

"(A) an estimated number of patients treated with each such drug, and the number of patients tracked in an observational registry under subsection (g) with respect to each

such drug, if applicable;

"(B) a discussion, at an aggregate level, of the types and amounts of data obtained through observational registries under subsection (g), such as patient treatments and uses, length of use, side effects encountered, relevant biomarkers, scan results, cause of death and how long the patient lived, and adverse drug effects;

"(C) a list of all such drugs for which an application for approval under this section, or an application for an extension of conditional approval under this section, has been submitted; and

"(D) the number of all applications granted and denied conditional approval under this section.

"(2) SPONSOR PARTICIPATION.—Not later than 180 days before the date on which the Secretary submits a report under paragraph (1), the sponsor of a drug conditionally approved under this section shall provide to the Secretary the information described in subparagraphs (A) and (B) of paragraph (1), as applicable.

"(3) NOTICE AUTHORITY.—The Secretary may notify sponsors of drugs conditionally approved under this section and observational registries under subsection (g) as necessary to complete a report under paragraph (1).".

(b) Conforming Amendment.—Section 505(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(a)) is amended by inserting ", or there is in effect a conditional approval under section 524C with respect to such drug" before the period.

(c) Reimbursement.—

(1) PRIVATE HEALTH INSURERS.—Section 2719A of the Public Health Service Act (42 U.S.C. 300gg–19a) is amended by adding at the end the following:

"(f) Coverage of Certain Drugs.—A group health plan or health insurance issuer offering group or individual health insurance coverage shall provide coverage for, and shall not impose any cost sharing requirements for, drugs conditionally approved under section 524C of the Federal Food, Drug, and Cosmetic Act for patients who have the disease or condition the drug is intended to treat.".

(2) FEDERAL HEALTH CARE PROGRAMS.—The requirement under subsection (f) of section 2719A of the Public Health Service Act (as added by paragraph (1)) shall apply with respect to coverage determinations under a Federal health care program (as defined in section 1128B(f) of the Social Security Act (42 U.S.C. 1320a–7b(f))) in the same manner such requirement applies under such subsection (f).

(3) CONFORMING AMENDMENT.—Section 1927(k)(2)(A)(i) of the Social Security Act (42 U.S.C. 1396r–8(k)(2)(A)(i)) is amended—

(A) by striking "or which" and inserting ", which"; and

(B) by inserting ", or which is conditionally approved under section 524C of such Act" before the semicolon.